(19) World Intellectual Property Organization International Bureau





(43) International Publication Date 28 July 2005 (28.07.2005)

PCT

(10) International Publication Number WO 2005/069192 A1

(51) International Patent Classification⁷: G01N 33/68

G06F 19/00,

(21) International Application Number:

PCT/US2005/001338

- (22) International Filing Date: 13 January 2005 (13.01.2005)
- (25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/536,451

13 January 2004 (13.01.2004) US

- (71) Applicants (for all designated States except US): TRAN-SKARYOTIC THERAPIES, INC. [US/US]; 700 Main Street, Cambridge, MA 02139 (US). NIH/NI-AID [US/US]; 6610 Rockledge Drive, Bethesda, MD 20892-6612 (US).
- (71) Applicants and
- (72) Inventors: GARMAN, Scott, C. [US/US]; NIH/NI-AID, Twinbrook 2, Room 109, 12441 Parklawn Drive, Rockville, MD 20852-1742 (US). GARBOCZI, David, N. [US/US]; NIH/NIAID, Twinbrook 2, Room 110, 12441 Parklawn Drive, Rockville, MD 20852-1742 (US). TRECO, Douglas, A. [US/US]; 87 Brentwood Road, Arlington, MA 02476 (US). SELDEN, Richard, F. [US/US]; 106 Bristol Road, Wellesley, MA 02482 (US). BOROWSKI, Marianne [US/US]; P.O. Box 1047, Glen, NH 03838 (US). KINOSHITA, Carol, M. [US/US]; 140 Davis Road, Bedford, MA 01730 (US).

- (74) Common Representative: TRANSKARYOTIC THER-APIES, INC.; 700 Main Street, Cambridge, MA 02139 (US).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, NA, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IS, IT, LT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

- with international search report
- before the expiration of the time limit for amending the claims and to be republished in the event of receipt of amendments

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: CRYSTAL STRUCTURE OF HUMAN α-GALACTOSIDASE

(57) Abstract: This invention pertains to the X-ray crystal structure of the human α -galactosidase glycoprotein. More specifically, the invention relates to crystallized compositions of human α -galactosidase and to crystallized complexes of human α -galactosidase and its catalytic product α -galactose. The invention further relates to a computer programmed with the structure coordinates of the human a galactosidase's active site wherein said computer is capable of displaying a three-dimensional representation of that active site. The invention also relates to methods for rational drug design based on the structural data for human α -galactosidase provided on computer readable media, as analyzed on a computer system having suitable computer algorithms.